Stem Cell Agency invests more than $44 million in treatments for stroke and fixing blood disorders in the womb

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June 29, 2017 San Francisco, CA Two projects aimed at helping people who have had a stroke, plus a therapy that hopes to correct a fatal blood disorder by operating on a baby in the womb, are among the programs approved today by the governing Board of the California Institute for Regenerative Medicine (CIRM).

The Board also honored C. Randal Mills, Ph.D., President and CEO of CIRM, and James Harrison, CIRM's General Counsel, both of whom are leaving the agency.

But first, the science. Every year around 800,000 Americans suffer a stroke; 130,000 people die; and for those who survive, strokes are the leading cause of serious, long-term disability in the US, representing an unmet medical need.

"Today the CIRM Board approved two very different methods, using different kinds of stem cells, to address this need," says Dr. Maria Millan, interim CEO and President of the agency. "By funding "multiple shots on goal" we believe that we have a better chance of finding a way to repair the damage caused by stroke and give people a better quality of life."

A team at SanBio was awarded almost $20 million to carry out a Phase 2 clinical trial using bone marrow-derived mesenchymal stem cells that have been modified to help people suffering from chronic disability following a stroke.

Stanford University's Dr. Gary Steinberg received $5.3 million to complete the pre-clinical studies needed to apply to the Food and Drug Administration (FDA) to test his neural cell therapy in a clinical trial.

Dr. Tippi MacKenzie, at the University of California, San Francisco, was awarded $12.1 million to treat babies in the womb who have alpha thalassemia major. This is a blood disorder that is only detected in the last few months of pregnancy and is almost always fatal. MacKenzie is using hematopoietic stem cells (HSCs), taken from the mother's bone marrow, and transplanting them into the baby before birth. The baby's immune system is able to tolerate the mother's cells, increasing the chances of a healthy birth, and improving the chances of having effective treatments after birth.

The Board also awarded Cellerant Therapeutics $6.86 million to conduct the studies needed to get FDA approval for a clinical trial to help people with acute myeloid leukemia (AML). This is a cancer that affects bone marrow and blood. The five-year survival rate for people over 60 with AML is just 26 percent.

Cellerant has developed a novel drug that will target Leukemic stem cells (LSC). It's believed that during chemotherapy LSCs are able to lie dormant and survive, and then later become active again allowing the cancer to return. It's hoped that targeting LSCs will reduce relapse rates and prolong survival.

The Board also approved investing more than $20 million in 13 projects in the Discovery Quest Awards Program. These are early stage research awards whose goal is to promote the discovery of promising new stem cell-based technologies that could be translated to enable broad use and ultimately improve patient care.

The successful applications are:

Application	Title	Institution	CIRM Committed Funding

DISC2-10088	Preclinical development of AAV vector-mediated in vivo hepatic reprogramming of myofibroblasts as a therapy for liver fibrosis	H. Willenbring, U.C. San Francisco	$1,638,389
DISC2-10110	Multipotent cardiovascular progenitor regeneration of the myocardium after MI	M. Mercola, Stanford University	$1,817,654
DISC2-10090	Human cardiac chip for assessment of proarrhythmic risk	K. Healy, U.C. Berkeley	$944,721
DISC2-10124	Targeted Gene Editing in the Treatment of X-Linked Hyper-IgM Syndrome	C. Kuo, U.C. Los Angeles	$1,665,908
DISC2-10061	Lgr5-mediated self-renewal in B cell selection and leukemia-initiation	M. Muschen, City of Hope	$2,186,520
DISC2-10120	Microenvironment for hiPSC-derived pacemaking cardiomyocytes	D. Lieu, U.C. Davis	$2,042,728
DISC2-10195	Identification and characterization of the optimal human neural stem cell line (hNSC) for the treatment of traumatic brain injury (TBI) 2.0	B. Cummings, U.C. Irvine	$1,671,213
DISC2-10182	Discovery of therapeutics for Huntington's Disease	A. Brivanlou, Rumi Scientific CA	$1,399,800

DISC2-10067	A tool for rapid development of clinical-grade protocols for dopaminergic neuronal differentiation of Parkinson's Disease patient-derived iPSCs	J. Cooper White, Scaled Biolabs Inc.	$677,160
DISC2-10129	Non-Toxic, Highly-Effective Bioinspired Cryoprotectants for On-Demand Stem Cell Therapies	X. Wei, X-Therma Inc.	$887,883
DISC2-10188	Immunization strategies to prevent Zika viral congenital eye and brain disease	V. Arumugaswami, Cedars-Sinai	$2,206,291
DISC2-10107	A Novel Approach to Eradicate Cancer Stem Cells	Y. Chen, City of Hope	$1,839,484
DISC2-10134	Platform Technolo gy for Pluripotent Stem Cell -Derived T cell Immunotherapy	G. Crooks, U.C. Los Angeles	$1,062,076

The meeting also marked a transition for the agency as the Board said farewell to Dr. Mills and CIRM's longtime General Counsel James Harrison.

After joining the stem cell agency in 2014 Dr. Mills launched CIRM 2.0, a radical overhaul of the way CIRM funds research, speeding up the time from application to funding (in the case of clinical trials from almost two years to just 120 days) and placing greater emphasis on people and partnerships. He also won unanimous Board approval for a five-year Strategic Plan that laid out ambitious goals for the agency including funding 50 new clinical trials by 2020.

Dr. Mills is leaving to be the next President and CEO of the National Marrow Donor Program® (NMDP)/Be The Match®. Maria Millan, M.D., will succeed Dr. Mills as interim President and CEO of CIRM pending further action by the agency's Presidential Search Subcommittee and full Board.

Jonathan Thomas, Ph.D., J.D., Chair of the Board, praised Dr. Mills saying: "We are deeply indebted to Randy for his extraordinary leadership. We knew from the moment he interviewed for the position that he was the right person for the job. He is that rare creature, a visionary who is able to inspire those around him and help turn his vision into reality. We shall miss him greatly but wish him and his family well."

Board member Sherry Lansing called him "a visionary and genius", Vice Chair Sen. Art Torres praised him for his compassion for patients and putting their needs above everything else.

Patient Advocates added their thanks and gratitude. Don Reed thanked him for making an already fine organization even better, working hard to advance treatments to paitents. Adrienne Shapiro presented him with the Heart of a Mother Award for his commitment to helping patients. Jake Javier wrote: "Something I will aways remember is how personal and genuine you were. I hope you got the chance to meet as many of the people you helped as possible because I know they would remember the same."

General Counsel James Harrison has been a vital part of CIRM since even before there was a CIRM. He helped draft Proposition 71, the voter-approved ballot initiative that created the agency in 2004. Since then he has helped guide the agency through numerous reviews and audits, and overhauled CIRM's programs and policies as part of CIRM 2.0. Harrison is leaving to take on a bigger role at the law firm of Remcho, Johansen & Purcell, LLP where he is a partner.

"It's hard to imagine CIRM without James," says Dr. Thomas. "He's been such an important part of the agency right from the very beginning. His wise counsel and calm leadership has helped us weather some challenging times and even though he is leaving us his legacy remains in everything we do."

Board member Jeff Sheehy said: "We would not be here without James, he wrote Prop 71, he organized the defense when we were sued by our opponents in the early days, through the various leadership challenges we had, all of the legal difficulties we had, James was there to guide us and it's been nothing short of extraordinary."

Harrison is succeeded by Scott Tocher, CIRM's Deputy General Counsel, who has been at the agency since 2005.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to **www.cirm.ca.gov.**

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